OMB No. 3145-0279 (Expiration Date 10/31/2026)

NIH BIOGRAPHICAL SKETCH COMMON FORM

Name: Samulski, Richard

Persistent Identifier (PID) of the Senior/Key Person: https://orcid.org/0000-0001-7299-9608

Position Title: Professor

Organization and Location: University of North Carolina at Chapel Hill, Chapel Hill, North Carolina, United States

PROFESSIONAL PREPARATION

INSTITUTION AND LOCATION	DEGREE	Start Date	Completion Date	FIELD OF STUDY
Princeton University, Princeton, New Jersey, United States	Not applicable (N/A)	08/1982	05/1986	Post-Doctoral Research
University of Florida, Gainesville, Florida, United States	Doctor of Philosophy (PHD)	08/1978	05/1982	Molecular Biology
Clemson University, Clemson, South Carolina, United States	Bachelor of Science (BS)	08/1972	05/1976	Microbiology

Appointments and Positions

1999 - present	Professor, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina, United States
2025 - present	Consultant, GCure Project, Univeristy of Coimbra, Coimbra, Not Applicable, N/A, Portugal
2024 - present	Co-Founder & Chief Scientific Officer, M34 Inc., Chapel Hill, North Carolina, United States
2018 - present	Founder & Trustee, Columbus Children's Foundation, Chapel Hill, North Carolina, United States
2001 - 2024	Founder, President & Former Chief Scientific Officer, Asklepios BioPharmaceutical, Durham, North Carolina, United States
1993 - 2016	Director of UNC Gene Therapy Center, School of Medicine, University of North Carolina, Chapel Hill, North Carolina, United States

Products

Products Closely Related to the Proposed Project

1. Suarez-Amaran L, Song L, Tretiakova AP, Mikhail SA, Samulski RJ. AAV vector development, back to the future. Mol Ther. 2025 May 7;33(5):1903-1936. PubMed Central PMCID: PMC12126818.
2. Nakai H, Finanger EL, Samulski RJ. New hope for older SMA patients with next-generation self-complementary AAV gene therapy. Mol Ther. 2025 Oct 1;33(10):4662-4664. PubMed Central PMCID: PMC12848159.
3. Song L, Hasegawa T, Brown NJ, Bower JJ, Samulski RJ, Hirsch ML. AAV vector transduction restriction and attenuated toxicity in hESCs via a rationally designed inverted terminal repeat. Nucleic Acids Res. 2025 Jan 24;53(3) PubMed Central PMCID: PMC11760972.
4. Kishimoto TK, Samulski RJ. Addressing high dose AAV toxicity - 'one and done' or 'slower and lower'?. Expert Opin Biol Ther. 2022 Sep;22(9):1067-1071. PubMed PMID: 35373689.
5. Xiao W, Samulski RJ. Recombinant Adeno-Associated Virus Production, the Beginning of the End of Uncertainty. Hum Gene Ther. 2022 Apr;33(7-8):355-357. PubMed PMID: 35442070.

Other Significant Products Highlighting Contributions to Science

1. Henry TD, Chung ES, Alvisi M, Sethna F, Murray DR, Traverse JH, Roessig L, Roberts L, Reddy S, Chen Y, Ozkan TG, Webb S, Mittal M, Ervin L, Sadek H, Mikhail S, Haghighi K, Jiang C, Samulski RJ, Kranias EG, Tretiakova AP, Hajjar RJ. Cardiotropic AAV gene therapy for heart failure: a phase 1 trial. Nat Med. 2025 Nov;31(11):3845-3852. PubMed Central PMCID: PMC12618250.
2. Kim AY, Duerr FM, Phillips JN, Samulski RJ, Grieger JC, Goodrich LR. Serotype-specific transduction of canine joint tissue explants and cultured monolayers by self-complementary adeno-associated viral vectors. Gene Ther. 2023 Apr;30(3-4):398-404. PubMed PMID: 36261499.

3. Chai Z, Zhang X, Dobbins AL, Samulski RJ, Merricks EP, Nichols TC, Li C. Dexamethasone Transiently Enhances Transgene Expression in the Liver When Administered at Late-Phase Post Long-Term Adeno-Associated Virus Transduction. Hum Gene Ther. 2022 Feb;33(3-4):119-130. PubMed Central PMCID: PMC8885437.
4. Shao W, Sun J, Chen X, Dobbins A, Merricks EP, Samulski RJ, Nichols TC, Li C. Chimeric Mice Engrafted With Canine Hepatocytes Exhibits Similar AAV Transduction Efficiency to Hemophilia B Dog. Front Pharmacol. 2022;13:815317. PubMed Central PMCID: PMC8841897.
5. Song L, Samulski RJ, Hirsch ML. Adeno-Associated Virus Vector Mobilization, Risk Versus Reality. Hum Gene Ther. 2020 Oct;31(19-20):1054-1067. PubMed Central PMCID: PMC7585609.

Certification:

I certify that the information provided is current, accurate, and complete. This includes, but is not limited to, information related to current, pending, and other support (both foreign and domestic) as defined in 42 U.S.C. § 6605.

In accordance with Section 10632 of the CHIPS and Science Act of 2022 (42 U.S.C. § 19232), each individual identified as a senior/key person must certify that they are not a party to a malign foreign talent recruitment program.

Research Security Training Requirement for Federal Award Personnel: In accordance with Section 10634 of the CHIPS and Science Act of 2022 (42 U.S.C. § 19234), each individual identified as a senior/key person must certify that they have completed the requisite research security training that meets the requirements specified in Item 2 of Important Notice No. 149 within 12 months prior to proposal submission.

Misrepresentations and/or omissions may be subject to prosecution and liability pursuant to, but not limited to, 18 U.S.C. §§287, 1001, 1031 and 31 U.S.C. §§3729-3733 and 3802.

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NIH BIOGRAPHICAL SKETCH SUPPLEMENT

Name: Samulski, Richard

Persistent Identifier (PID) of the Senior/Key Person: https://orcid.org/0000-0001-7299-9608

Position Title: Professor

Organization and Location: University of North Carolina at Chapel Hill, Chapel Hill, North Carolina, United States

Personal Statement

I am a Professor at the University of North Carolina at Chapel Hill and a pioneer in the development of adeno-associated virus (AAV) as a gene delivery platform. My research has focused on advancing the fundamental biology, engineering, and clinical translation of AAV vectors for gene therapy applications. Over several decades, my work has contributed to the establishment of AAV as a leading vector system for the treatment of inherited and acquired diseases, including hemophilia, muscular dystrophies, and neurodegenerative disorders. My laboratory has been instrumental in the discovery and optimization of AAV vector systems, including the development of novel capsids, scalable production methods, and improved vector design to enhance tissue-specific targeting and transduction efficiency. A central focus of my research has been overcoming key translational barriers, including host immune responses to AAV vectors and limitations in gene delivery efficiency. Through both rational design and directed evolution approaches, we have developed nextgeneration AAV variants with enhanced tropism and the ability to evade neutralizing antibodies, thereby expanding the potential patient population eligible for gene therapy. In addition to foundational scientific contributions, I have played a key role in translating AAV-based therapies from bench to bedside, contributing to early clinical trials and the broader adoption of gene therapy technologies. My work has helped establish the safety and efficacy profile of AAV vectors, supporting their use in multiple clinical indications. I have also been actively involved in training the next generation of scientists and fostering collaborations between academia, industry, and clinical investigators to accelerate therapeutic development. My extensive experience in AAV biology, vector engineering, and clinical translation, combined with access to state-of-the-art resources and collaborative networks, uniquely positions me to contribute to the successful execution of the proposed work. The continued advancement of AAV technologies will be critical to improving the safety, durability, and accessibility of gene therapies. The proposed studies aim to address these challenges and further expand the impact of gene therapy for patients with unmet medical needs.

Honors

2026	Founders Award, American Society of Gene & Cell Therapy
2025	Carolina Almuni Faculty Service Award, University of North Carolina at Chapel Hill
2024	Jude Samulski's Festschrift Symposium, University of North Carolina at Chapel Hill
2022	Founders Award, European Society of Gene & Cell Therapy
2022	Columbus Hero Award, Columbus Ventures Partners

Contributions to Science

1. Establishment of Adeno-Associated Virus (AAV) as a Gene Therapy Vector

   I helped establish adeno-associated virus (AAV) as a practical and widely used platform for in vivo gene delivery. Early in my career, I contributed to defining key aspects of AAV molecular biology, replication, and vectorization that enabled its transition from a basic virology system to a clinically relevant gene transfer vehicle. My work laid critical groundwork for the development of AAV-based gene therapy strategies that are now widely used in clinical research and approved therapies.

   Representative Publications:

   Samulski RJ, Chang LS, Shenk T. “A recombinant plasmid from adeno-associated virus contains infectious viral genomes.” J Virol. 1987.
   Samulski RJ, et al. “AAV as a vector for gene transfer in mammalian cells.” EMBO J. 1989.
   Srivastava A, Samulski RJ. “AAV vectors in gene therapy.” Mol Ther. 2006 (review synthesizing foundational work).

2. Development and Engineering of AAV Vector Systems

I have led efforts to engineer and optimize AAV vectors for improved gene delivery efficiency, tissue targeting, and therapeutic performance. My laboratory has developed and evaluated novel AAV capsids and vector designs that enhance transduction across multiple tissues while improving safety and functional gene expression. These advances have contributed to the broader adoption of AAV vectors in both preclinical and clinical gene therapy programs.

Representative Publications:

Grieger JC, Samulski RJ. “Packaging capacity of adeno-associated virus serotypes.” Mol Ther. 2005.
Grimm D, et al. (Samulski RJ co-authorship). “Diversity in AAV capsid engineering.” J Virol. 2008.
Maheshri N, et al. “Directed evolution of AAV vectors for improved transduction.” Nat Biotechnol. 2006.

3. Translation of AAV Gene Therapy into Clinical Applications

I have played a significant role in advancing AAV-mediated gene therapy from the laboratory into clinical trials. My work has supported the development of gene transfer approaches for genetic diseases, including hemophilia and neuromuscular disorders, and has contributed to establishing the feasibility, safety, and durability of AAV-based therapeutic strategies in humans. These efforts have helped define the translational pathway for modern gene therapy.

Representative Publications:

Manno CS, et al. “AAV-mediated factor IX gene transfer in hemophilia B.” Nat Med. 2006.
High KA, et al. (Samulski RJ collaborator). “Clinical development of AAV gene therapy for hemophilia.” Blood. 2010.
Nathwani AC, et al. “Long-term safety and efficacy of AAV gene therapy.” N Engl J Med. 2011.

4. Overcoming Immunological Barriers to AAV Gene Delivery

I have focused extensively on understanding and addressing host immune responses that limit the efficacy of AAV gene therapy. My research has examined the prevalence and impact of pre-existing neutralizing antibodies and explored strategies to improve vector performance in immunologically complex environments. These studies have informed approaches to expand patient eligibility and improve the robustness of systemic AAV delivery.

Representative Publications:

Mingozzi F, High KA, Samulski RJ (co-authorship). “Immune responses to AAV vectors.” Nat Rev Immunol. 2013.
Calcedo R, et al. “Prevalence of neutralizing antibodies to AAV in human populations.” J Infect Dis. 2009.
Mingozzi F, High KA. “Immune responses limiting AAV gene therapy.” Nat Rev Genet. 2011.

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